

CLEAN COPY OF CLAIMS:



Atty's Dkt No.: 018743/027 6322
Client Ref. No.: CHOP-0004DF

RECEIVED

NOV 29 2002

TECH CENTER 1600 2900

1. A composition comprising a virus, said virus comprising a recombinant adeno-associated virus vector comprising at least two adeno-associated virus inverted terminal repeats, a promoter/regulatory sequence, isolated DNA encoding Factor IX and accompanying 5' and 3' untranslated regions and a transcription termination signal.
2. The composition of claim 1, further comprising a portion of intron 1 of a Factor IX gene.
3. The composition of claim 2, wherein said portion of intron 1 of a Factor IX gene is from about 0.3 kb to about 1.7 kb in length.
4. The composition of claim 1, wherein said isolated DNA encoding Factor IX comprises a mutation which renders Factor IX encoded thereby incapable of binding to collagen IV.
5. The composition of claim 4, wherein said mutation encodes an alanine residue in place of lysine in the fifth amino acid position from the beginning of mature F.IX.
6. The composition of claim 1 further comprising a pharmaceutically acceptable carrier.
7. The composition of claim 1, wherein said promoter/regulatory sequence is selected from the group consisting of the cytomegalovirus immediate early promoter/enhancer, the skeletal muscle actin promoter and the muscle creatine kinase promoter/enhancer.

8. The composition of claim 1, wherein said transcription termination signal is the SV40 transcription termination signal.
9. A kit including the vector of claim 1, and instructions for using said kit.
10. A composition comprising a recombinant adeno-associated virus vector comprising at least two adeno-associated virus inverted terminal repeats, a promoter/regulatory sequence, isolated DNA encoding Factor IX, wherein said isolated DNA encoding Factor IX comprises a mutation which renders Factor IX encoded thereby incapable of binding to collagen IV, and accompanying 5' and 3' untranslated regions and a transcription termination signal.
11. The composition of claim 10, wherein said mutation encodes an alanine residue in place of lysine in the fifth amino acid position from the beginning of mature F.IX.
12. The composition of claim 10, further comprising a portion of intron 1 of a Factor IX gene.
13. The composition of claim 12, wherein said portion of intron 1 of a Factor IX gene is from about 0.3 kb to about 1.7 kb in length.
14. The composition of claim 1 further comprising a pharmaceutically acceptable carrier.
15. The composition of claim 1, wherein said promoter/regulatory sequence is selected from the group consisting of the cytomegalovirus immediate early promoter/enhancer, the skeletal muscle actin promoter and the muscle creatine kinase promoter/enhancer.

16. The composition of claim 1, wherein said transcription termination signal is the SV40 transcription termination signal.
17. A kit including the vector of claim 1, and instructions for using said kit.